



Pathways to a Cure

The CAA Newsletter

MESSAGE FROM THE DIRECTOR

It is with great anticipation that we warmly welcome you to our first CAA newsletter, *Pathways to a Cure!* It is our hope that this newsletter will inform our valued research volunteers and their families about the latest science and research advances at the J. Philip Kistler MGH Stroke Research Center and other leading research centers around the globe.

This is undoubtedly one of the most exciting times in the history of the hemorrhagic stroke research field as we begin a new major trial to treat CAA (*please see our lead article*). Having recently attended the International CAA Conference in Leiden, Netherlands, I am happy to report a rapidly growing international consensus on the direction of CAA research and treatment. We are truly redefining our ability to indentify patient populations and offer them new and exciting critical research opportunities.

Our newsletter is here to serve you, and we welcome your thoughts, hopes and opinions. We look forward to hearing from you and providing you with a source of interactive and helpful knowledge on CAA research.

Sincerely,
Steve Greenberg, MD, PhD



A New Experimental Treatment for CAA

The JP Kistler Stroke Research Center has partnered with Pfizer Pharmaceuticals to conduct a novel drug treatment trial for Cerebral Amyloid Angiopathy (CAA). The experimental drug, called Ponezumab (PF-04360365), is an antibody believed to reduce the amount of harmful amyloid protein in the brain.

Ponezumab is administered through an IV directly into the blood stream. The drug then travels to the brain, where it binds to and removes the amyloid protein. Once free from the brain, the amyloid can be discarded by the body's normal metabolic processes.

In CAA, amyloid protein coats the walls of the brain's small blood vessels. This amyloid protein accumulation can cause blood vessel dysfunction, and ultimately lead to bleeding in the brain. It is thought that by removing amyloid protein, the course of CAA may be slowed.

The Ponezumab drug trial is a multicenter effort, with Massachusetts General Hospital projected to be the leading site. In order to test Ponezumab's efficacy, the trial is structured so that 2 of every 3 subjects will receive Ponezumab. Participants are asked to attend 11 study visits spanning 8 months, during which they will receive 3 administrations of the study treatment. Each participant will have functional Magnetic Resonance Imaging (fMRI)

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A new experimental treatment

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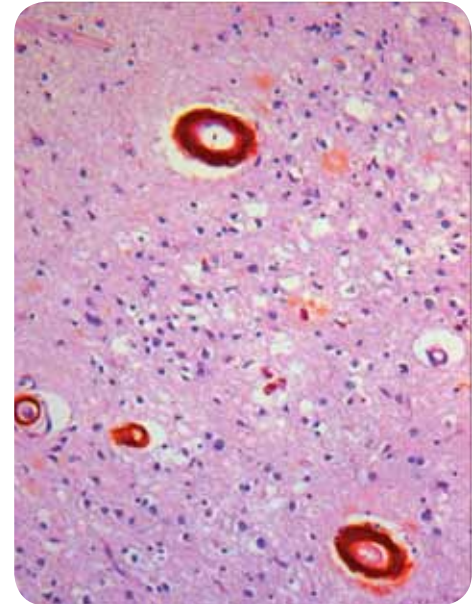
scans to detect any changes in blood vessel function in the brain.

Participants will also be monitored via electrocardiography (ECGs), physical exams, and cognitive testing.

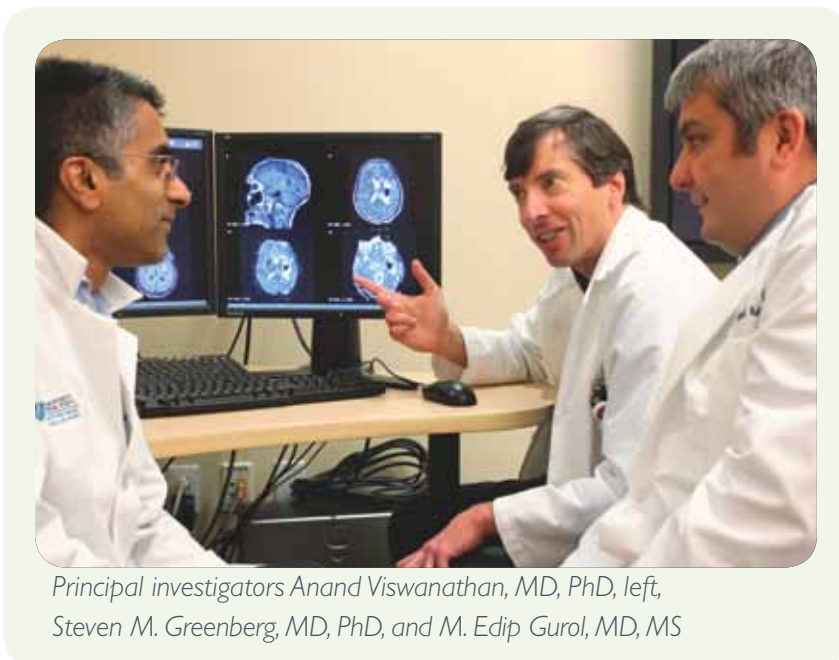
Dr. Steven Greenberg, Director of the Stroke Center, elaborates on the trial's significance, "This trial has the potential to change everything. We have an exciting opportunity to test a completely novel approach to slowing the course of CAA. This is the first time somebody has used antibodies to clear the amyloid that deposits in the blood vessel walls in people with CAA. This is also the first

use of functional MRI as a marker of how blood vessel walls are responding, and whether we can prevent worsening or actually cause improvement in how the blood vessels function in people with CAA." Although we do not know what the results of this important trial will show, it marks the genesis of a truly exciting and hopeful time for CAA patients and their loved ones.

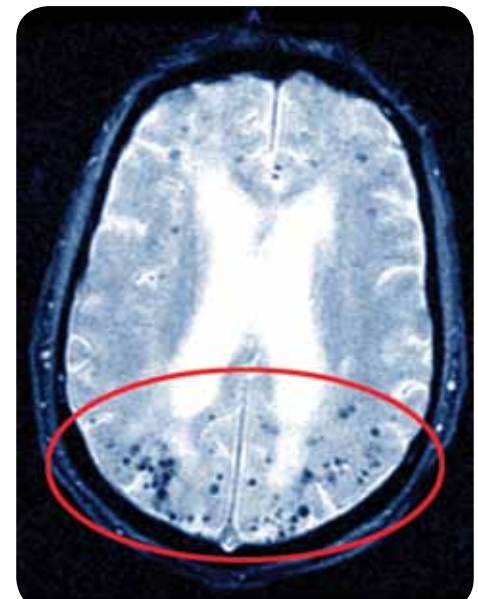
If you would like to learn more about the treatment trial, please call Clinical Research Coordinator, Thomas Gomes at 617-726-2941 or email Tgomes3@partners.org for more information.



In CAA, amyloid protein coats the walls of brain vessels (*above*) and can cause bleeding in the brain. The MRI image (*below*) shows small spots of bleeding in the brain, mainly located in the occipital lobes (*bottom of the image*).



Principal investigators Anand Viswanathan, MD, PhD, left, Steven M. Greenberg, MD, PhD, and M. Edip Gurol, MD, MS



PATIENT SPOTLIGHT:

Veteran CAA Research Participant Raises Funds and Awareness

In each edition of *Pathways to a Cure*, we will highlight a research participant who has inspired us. We have been touched by our participants' fervent commitment to research. In this edition we spoke with veteran research participant Robert Jones and his daughter Cindy Jones, who fundraise and spread awareness for CAA.

What motivates you to be so involved in CAA research?

Cindy: CAA is such a rare condition, so it is important for people to hear and learn about it. Family members may be experiencing it, and they do not even know what it is. When my dad first started showing signs we did not know what it was either.

Bob: The best thing I can do is be with a great team. I am very well greeted and taken care of when I come here. The staff here has a great attitude. My time is appreciated, and that is very apparent here.

Could you tell us a little about your fundraising events?

Bob: We certainly had a party! Our fundraiser was held at a minor league baseball stadium. This stadium is the hit of the town and we just thought that would be a neat place to have a fundraiser.

What would you like to say to people with CAA and their families?

Cindy: The most important message I have is the person who has CAA needs love, compassion, and support. You are not alone! There are other people going through the same thing. Reach out to the experts, and ask questions.



Robert (Bob) and daughter, Cindy Jones

Cindy: I started fundraising because of the powerless position I am in. There is nothing I can do about the disease, however, I can fight through this difficult diagnosis by fundraising, and spreading awareness and education so people can learn and grow. It is a labor of love.

If you would like to contact Bob and Cindy Jones with questions or comments or are interested in arranging your own fundraiser, please call Clinical Research Coordinator Anne Reed at 617-643-2782 or email akreed@partners.org for more information.

J. Philip Kistler MGH Stroke Research Center

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www.angiopathy.org

Contact Information:

Please send your comments, questions and recommendations to:

pathwaystoacure@partners.org

For more information on our CAA research and for FAQs, please visit:

www.angiopathy.org

To donate to Dr. Greenberg's CAA research center please visit:

<http://angiopathy.org/support.html>

If you prefer to make a donation via check, you can do so by making your check out to "Massachusetts General Hospital" with

memo "Fund 1200-028184 and mailing it to:

MGH Development Office

Attn: Shawn Fitzgibbons

165 Cambridge Street, Suite 600
Boston, MA 02114



Thank you for your commitment to CAA research!

From all of us at The J. Philip Kistler MGH Stroke Research Center